

## **CIRM, the UK and Canada Award more than \$250 Million to Accelerate the Pace of Bringing Stem Cell Therapies to the Clinic**

Posted: October 28, 2009

### **Novel funding mechanism speeds the path of research**

Los Angeles, Calif., October 28, 2009 – The California Institute for Regenerative Medicine, the state stem cell agency, and two international partners awarded more than \$250 million to 14 multidisciplinary teams of researchers in California, the UK and Canada to develop stem cell-based therapies for 11 diseases. The Disease Team Research Awards include approximately \$8 million from the Medical Research Council, UK, and approximately \$35 million from the Cancer Stem Cell Consortium, Canada, to fund the international portions of the collaborations.

CIRM's 29-member Governing Board voted to approve funding for the four-year grants, which mark the first CIRM funding explicitly expected to result in a filing with the FDA to begin a clinical trial. The Disease Team Research Awards fund research teams that include basic scientists and clinicians from both academia and industry. These collaborations speed the process of establishing clinical trials by insuring that clinically relevant issues are considered early and avoiding potential safety issues being discovered late in the process.

CIRM President Alan Trounson said the pace of the Disease Team projects stands in contrast to the decade or more that's usually required to reach clinical trials. "Scientists have talked for years about the need to find ways to speed the pace of discovery. By encouraging applicants to form teams composed of the best researchers from around the world we think CIRM will set a new standard for how translational research should be funded," he said.

Each team will be actively managed by CIRM and the agency's international partners for those teams with cross-border collaborations. Decisions to move forward with the project will be made at key points in the development cycle.

"This unique partnership is another opportunity for the people of California to lead the way in this important research and advance potentially life-saving science," said Governor Schwarzenegger. "These grants will help unite some of the best scientists throughout the world, including right here in California, to find new therapies and cures for people suffering from chronic and life-debilitating diseases. I am proud California remains at the forefront of this innovative research and I look forward to the results of this international collaboration."

"This initiative is bringing together the leading minds in cancer and stem cell research from Canada and California," said Dr. Morag Park, Scientific Director of the Institute of Cancer Research, part of the Canadian Institutes of Health Research (CIHR), the Government of Canada's health research agency. "CIHR, in conjunction with Genome Canada and through the Cancer Stem Cell Consortium, is proud to fund Canadian Scientists in this cross-border collaboration that will engage scientists from many disciplines, combine resources, technologies and knowledge to find more effective treatments for leukemia and solid cell tumours."

Sir Leszek Borysiewicz, Chief Executive of the Medical Research Council: "The partnerships that have been established between the UK and CIRM have brought us closer to delivering the promise of stem cell treatments for debilitating conditions. We hope these projects will accelerate treatments to early clinical trials, eventually leading to a direct benefit for people suffering from age-related macular degeneration, which up until now has been regarded as incurable and also acute myeloid leukaemia. The MRC has led the way for UK translational researchers and together with our partners at CIRM we look forward to realising the full potential of stem cell research"

Other diseases being targeted by the teams include HIV/AIDS, type 1 diabetes, damage from heart attack, sickle cell anemia, amyotrophic lateral sclerosis also known as Lou Gehrig's disease, and epidermolysis bullosa, a hereditary life-threatening condition of the skin's connective layer. The 14 awards will go to seven not-for-profit institutions and one for-profit institution. The award to the one for-profit grantee will take the form of a loan.

"CIRM's loan program will recycle money back into future awards and leverage the voter's commitment to the field," said Robert Klein, Chair of the CIRM Governing Board. "In providing stem cell funding in the form of loans, CIRM is able to fund more science and make a more significant impact on the speed of bringing new stem cell-based therapies to the people of California and the world."

## Other ICOC Business

The board also voted to approve an update to CIRM's on-going strategic plan. The current plan, approved in 2006, anticipated a slower pace of research toward potential clinical applications. The revision proposes an increased emphasis on moving safety tested candidate therapeutics to the clinic and encourages closer ties to industry and national and international collaborators to meet those goals.

### Approved Disease Team projects:

(See the Disease Team media materials for more information about the funded teams.)

Grant number	Investigator	Institution	Intl. Collaborator	Total CIRM Funding
DR1-01421	Karen Aboody	City of Hope National Medical Center		\$18,015,429
	Co-PIs: Jana Portnow	City of Hope National Medical Center		
	Larry Couture	City of Hope National Medical Center		
	The group proposes to treat brain tumors using neural stem cells that are genetically modified to carry a tumor-killing drug.			
DR1-01423	Emmanuel Baetge	Novocell, Inc		\$19,999,937
	Co-PI: Jeffrey Bluestone	University of California, San Francisco		
	The group proposes to treat people with type 1 diabetes by implanting insulin-producing cells generated from human embryonic stem cells.			
DR1-01426	Mitchel Berger	University of California, San Francisco		\$19,162,435
	Co-PIs: Webster Cavenue	Ludwig Institute for Cancer Research		
	Evan Snyder	Burnham Institute for Medical Research		
	The group proposes to treat brain tumors using neural stem cells that are genetically modified to carry a tumor-killing drug.			
DR1-01430	Dennis Carson	University of California, San Diego	Canada	\$19,999,826
	Co-PI: Catriona Jamieson	University of California, San Diego		
	International Partner: John Dick	University Health Network		
	The group intends to develop six drugs – three monoclonal antibodies and three small molecules – to destroy leukemia stem cells.			
DR1-01431	Irvin Chen	University of California, Los Angeles		\$19,999,580
	Co-PI: Geoff Symonds	Calimmune, Inc		
	This group proposes to treat HIV/AIDS using an RNA interference approach to modify the patient's blood-forming stem celis. When transplanted back, those cells will produce T cells that are resistant to HIV infection.			
DR1-01444	Mark Humayun	University of Southern California	MRC	\$15,904,916
	Co-PIs: David Hinton	University of Southern California		
	Dennis Clegg	University of California, Santa Barbara		
	International Partner: Peter Coffey	University College London-Institute of Ophthalmology		
	The group intends to treat macular degeneration using transplant retinal cells derived from human embryonic stem cells.			
DR1-01452	Donald Kohn	University of California, Los Angeles		\$9,212,365
	Co-PIs: Thomas Coates	Children's Hospital of Los Angeles		
	Victor Marder	University of California, Los Angeles		
	The group proposes to treat sickle cells disease using a gene therapy approach to modify the patient's blood-forming stem cell so that they produce normal red blood cells.			
DR1-01454	Alfred Lane	Stanford University		\$11,709,574
	Co-PIs: Anthony Oro	Stanford University		
	Marius Wernig	Stanford University		
	The group proposes to treat the skin disease epidermolysis bullosa using genetically modified iPS cells created from the patient's own skin cells.			
DR1-01461	Eduardo Marban	Cedars-Sinai Medical Center		\$5,560,232
	The group intends to repair heart tissue damaged by heart attack using stem cells taken from the patient's own heart.			
	Samuel Pfaff	The Salk Institute for Biological Studies		

DR1-01471	Co-PIs: Lawrence Goldstein	University of California, San Diego		\$15,644,881
	Don Cleveland	Ludwig Institute for Cancer Research		
	The group intends to treat people with Amyotrophic lateral sclerosis by implanting precursor astrocyte cells derived from human embryonic stem cells.			
DR1-01477	Dennis Slamon	University of California, Los Angeles	Canada	\$19,979,660
	Co-PIs: Garry Nolan	Stanford University		
	Michael Press	University of Southern California		
	International Partner: Tak Wah Mak	University Health Network		
	The group proposes to develop drugs that destroy the cancer stem cells in solid tumors.			
DR1-01480	Gary Steinberg	Stanford University		\$20,000,000
	Co-PI: Stanley Carmichael	University of California, Los Angeles		
	The group intends to treat stroke using implanted neural stem cells derived from human embryonic stem cells.			
DR1-01485	Irving Weissman	Stanford University	MRC	\$19,999,996
	Co-PIs: Ravindra Majeti	Stanford University		
	Beverly Mitchell	Stanford University		
	International Partner: Paresh Vyas	Weatherall Institute of Molecular Medicine, Oxford University		
	The group intends to generate a monoclonal antibody that destroys leukemia stem cells.			
DR1-01490	John Zaia	City of Hope National Medical Center		\$14,583,187
	Co-PIs: Paula Cannon	University of Southern California		
	David DiGiusto	Beckman Research Institute of City of Hope		
	This group proposes to treat HIV/AIDS using a gene therapy approach to modify the patient's blood-forming stem celis. When transplanted back, those cells will produce T cells that are resistant to HIV infection.			
Total funding				\$229,772,018

#### All CIRM funded grants to-date:

Institution	Total Grants	Funds (Requested & Awarded)
Stanford University	42	162,979,744
University of California, Los Angeles	32	121,991,821
University of California, San Francisco	30	102,971,301
University of Southern California	18	68,096,825
University of California, San Diego	25	65,583,177
University of California, Irvine	22	59,757,564
University of California, Davis	15	49,088,145
Sanford Consortium for Regenerative Medicine	1	43,000,000
City of Hope National Medical Center	7	36,730,319
The Salk Institute for Biological Studies	13	35,051,452
University of California, Berkeley	10	34,626,605
Scripps Research Institute	11	27,560,249
Novocell, Inc.	4	26,281,356

Buck Institute for Age Research	4	25,429,364
Burnham Institute for Medical Research	15	23,134,219
The J. David Gladstone Institutes	13	22,633,003
University of California, Santa Cruz	9	19,383,633
Childrens Hospital Los Angeles	7	14,219,310
University of California, Merced	5	8,494,301
University of California, Santa Barbara	5	8,490,842
University of California, Riverside	4	6,055,762
Cedars-Sinai Medical Center	2	5,607,118
BioTime, Inc.	1	4,721,706
The Jackson Laboratory West	1	3,841,240
The Parkinson's Institute	1	3,701,766
San Diego State University	2	3,441,860
Scripps Health	1	3,118,431
Ludwig Institute for Cancer Research	3	2,473,053
California Institute of Technology	1	2,071,823
San Jose State University	1	1,733,760
California State University, Channel Islands	1	1,733,406
California State University, San Marcos	1	1,732,164
Pasadena City College	1	1,727,991
San Francisco State University	1	1,713,558
Humboldt State University	1	1,616,363
California State Polytechnic University, Pomona	1	1,436,797
California Polytechnic State University, San Luis Obispo	1	1,396,509
California State University, Long Beach	1	1,337,700
California State University, Sacramento	1	1,321,440
VistaGen Therapeutics, Inc.	1	971,558
Gamma Medica-Ideas, Inc.	1	949,748
Vala Sciences, Inc.	1	906,629
Invitrogen Corporation	1	869,262
Fluidigm Corporation	1	749,520
Human BioMolecular Research Institute	1	714,654

Childrens Hospital Oakland Research Institute	1	55,000
<b>Grand Total</b>	<b>321</b>	<b>\$1,011,502,048</b>

### About CIRM

CIRM was established in November, 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided \$3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research opportunities. For more information, please visit [www.cirm.ca.gov](http://www.cirm.ca.gov).

### The Cancer Stem Cell Consortium

The Cancer Stem Cell Consortium is a not-for-profit corporation that was incorporated in 2007 to coordinate an international strategy for cancer stem cell research and related translational activities. The strategy will allow the biomedical community to move quickly and effectively from discoveries to application in the clinic; establish partnerships among organizations from Canada, California and other jurisdictions to accelerate and synergize research and translation opportunities related to cancer stem cells; and secure investments from governments, private foundations and the private sector for sustained and stable research funding. Current Consortium members include: the Canada Foundation for Innovation, the Canadian Institutes of Health Research, Genome Canada, the Michael Smith Foundation for Health Research, the National Research Council Canada, the Ontario Institute for Cancer Research and the Stem Cell Network.

### About the MRC

The Medical Research Council supports the best scientific research to improve human health. Its work ranges from molecular level science to public health medicine and has led to pioneering discoveries in our understanding of the human body and the diseases which affect us all. MRC is the UK's major funder of stem cell research and has a key role in delivering Government's expectations in this area. [www.mrc.ac.uk](http://www.mrc.ac.uk)

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